

## What Study Participants Can Expect

People who are eligible for the study and decide to participate will receive:

- The investigational medication or placebo and all study-related care at no cost
- Care and close monitoring during the study from doctors and staff who understand their condition
- The option to join the open-label extension (OLE) study after completing the study where all participants will receive the investigational medication
- Reimbursement for expenses related to study participation such as travel, meals, etc. may be available

To help make an informed decision on whether or not to take part in this study, potential participants will go through a process called informed consent. The informed consent process will review what is involved in participating in the study and the potential risks and benefits. A participant can leave the study at any time, for any reason, without penalty.



To learn more about this study, or to find out if you, your child, or a loved one may qualify, please visit the Skyline Study website at [skyline-study.com](https://skyline-study.com) or [clinicaltrials.takeda.com](https://clinicaltrials.takeda.com).

# New Horizons

**A Clinical Research Study for Children, Teens, and Young Adults Diagnosed With Dravet Syndrome – The Skyline Study**



## A Person May Qualify for the Skyline Study If He/She:

- Is 2 to 21 years old
- Has been diagnosed with Dravet syndrome
- Has had 12 or more convulsive seizures over 12 weeks prior to screening
- Has been unable to control seizures with at least 1 anti-seizure medication in the past

Study staff will determine eligibility based on additional study criteria.

## What Is Dravet Syndrome?

Dravet syndrome (DS) is a rare epilepsy syndrome most commonly caused by a genetic mutation. The syndrome usually begins during infancy or early childhood and is characterized by prolonged focal seizures that can evolve into other seizure types. Those with DS often have a resistance to anti-seizure medications, leading to limited availability of treatment options.



## About the Skyline Study

The Skyline Study is a Phase 3 clinical research study of an investigational medication for children, teens, and young adults diagnosed with Dravet syndrome who are currently taking 0 – 4 anti-seizure medications to manage symptoms and are still experiencing seizures. The investigational medication, soticlestat, works differently than currently approved medications and is being tested as an add-on therapy in the clinical research study.

The goal of the Phase 3 clinical research study is to assess the effectiveness, safety and tolerability of the investigational medication when taken with other anti-seizure treatments. In previous clinical research studies, the investigational medication has been shown to reduce the number of seizures in study participants.

During the study, participants will be split into two groups: one will receive the study drug and one will receive placebo (looks like the study drug but contains no active ingredients). After completing the study, participants can join the open-label extension (OLE) study where every participant will receive the study drug.

## Study Participation Involves:

- Up to 5 planned in-person study visits; the rest of the study visits can be conducted virtually
- Approximately 25 weeks of study participation

## What Will Occur During the Study?

- **Screening (4–6 weeks):** During screening, it will be determined whether you, your child, or a loved one qualifies for the study.
- **Titration Period (4 weeks):** During the first visit of the four-week titration period, participants will be randomized into one of two groups. One group will receive the study drug, and one group will receive placebo. The placebo will look like the study drug but have no active ingredients. Titration is the process of adjusting a dose of medication to determine the dose a person can tolerate. During the four weeks, participants will receive the study drug or placebo at increased doses. The dose will be increased throughout the titration period as long as participants don't have any tolerability issues. If participants can't tolerate the minimum dose, they will discontinue from the study.
- **Maintenance Period (12 weeks):** The dose a participant receives at the end of the titration period will be taken throughout the 12-week maintenance period. Eligible participants may have the option to join the open-label extension (OLE) study after completing the maintenance period (based on selection criteria for the OLE study). During the OLE study, all participants (including those who received placebo) will take the investigational drug.
- **Follow-up (3 weeks):** Participants who are not joining the OLE study will taper off the study drug over one week and complete a safety follow-up visit or phone call approximately two weeks after receiving their last dose of the study drug.

Throughout participation in the study, all study-related visits, tests, and study medication will be provided at no cost. Reimbursement for expenses related to study participation such as travel, meals, etc. may also be available.